# Solid Biosciences Outlines Key Priorities for Advancing Diversified Neuromuscular and Cardiac Development Pipeline and Establishing Leadership in Precision Genetic Medicines

January 15, 2025

*- **Duchenne**: Dosed four patients in INSPIRE DUCHENNE clinical trial; SGT-003 has been well tolerated in all patients with no SAEs observed; initial three patient data expected Q1 2025 -*

*- **FA**: First-in-human clinical study of SGT-212 utilizing a dual route of administration to treat Friedreich's ataxia (FA) anticipated in 2H 2025 following recent FDA IND clearance -*

*- **CPVT**: Submission of IND for SGT-501 on track for 1H 2025; the submission will expand clinical-stage portfolio to three unique candidates in diverse indications with significant unmet need -*

*- **Cash**: Company enters 2025 with approximately $148.9 million in cash and investments[1] as of 12/31/2024; expected to fund key strategic priorities into 2026 -*

CHARLESTOWN, Mass., Jan. 15, 2025 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) (the "Company" or "Solid"), a life sciences company developing precision genetic medicines for neuromuscular and cardiac diseases, will provide a corporate update outlining the Company's expanded clinical-stage pipeline and 2025 objectives as it completes its transformation into a multi-program leader in the development of precision genetic medicines. Bo Cumbo, President and CEO, and Gabriel Brooks, M.D., Chief Medical Officer, will provide the update at the 43rd Annual J.P. Morgan Healthcare Conference on Wednesday, January 15, 2025, at 3:45 p.m. PT (6:45 p.m. ET).

"At the time I joined Solid Biosciences there was a singular focus on genetic treatments for Duchenne muscular dystrophy," said Bo Cumbo, President and CEO of Solid Biosciences. "In the last two years, we have broadened the pipeline to include gene therapy treatments for other devastating genetic neuromuscular and cardiac diseases, entered the clinic with our next generation gene therapy for Duchenne, opened a second IND with an industry first dual route of administration gene therapy to treat Friedreich's ataxia, and continued to make advancements in next-generation capsid technologies, novel promoters, immunomodulation techniques, and manufacturing excellence. We are committed to innovating and building a true 'next generation' genetic medicines company, while maintaining Solid's historic focus on the needs of the patient."

He continued: "With two clinical stage assets and a third IND submission anticipated in 1H 2025, Solid Biosciences stands on the threshold of realizing that vision: a platform poised to create transformative genetic medicines for patients and create value for our shareholders."

Highlights from the presentation to be given at the J.P. Morgan Healthcare Conference include:

**Neuromuscular Pipeline**
*SGT-003 for Duchenne muscular dystrophy (Duchenne)*

- SGT-003 continues to be well tolerated in the first four patients dosed in the ongoing INSPIRE DUCHENNE clinical trial.
  - Patient enrollment and dosing under the expanded INSPIRE DUCHENNE protocol is ongoing.
  - Six clinical sites are activated in North America (five in the U.S., one in Canada).
  - The U.K. Medicines and Healthcare products Regulatory Agency (MHRA) cleared the INSPIRE DUCHENNE clinical trial application (CTA) with initial site activation expected in 2H 2025.
  - Solid expects to report data from the first three patients dosed in Q1 2025.

*SGT-212 for Friedreich's ataxia (FA)*

- As announced on January 7, 2025, the FDA has cleared the IND for SGT-212 for the treatment of cardiac and CNS manifestations of FA. SGT-212 is the first and only gene therapy to utilize a dual route of administration to treat FA.
  - FA is a degenerative disease caused by insufficient levels of frataxin protein. SGT-212 is an AAV-based FA gene therapy candidate designed to deliver full-length frataxin via direct intradentate nuclei (IDN) infusion into the cerebellum to treat neurologic manifestations and systemic intravenous (IV) infusion to target cardiac and systemic manifestations.

- The Company expects to initiate a first-in-human, open-label, Phase 1b clinical trial of SGT-212 in 2H 2025. The study will enroll non-ambulatory and ambulatory adult patients living with FA across up to three cohorts and will evaluate the safety and tolerability of contemporaneous systemic and bilateral IDN administration of SGT-212.

**Cardiac Pipeline**
*SGT-501 for Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)*

- IND-enabling Good Laboratory Practice (GLP) toxicology studies in non-human primates (NHP) are underway, with the in-life portion of the six-month GLP toxicology study expected to be completed in Q1 2025.
- The Company anticipates submitting an IND for SGT-501 for the treatment of CPVT in 1H 2025.

*SGT-601 for TNNT2 Thin Filament Cardiomyopathy*

- Preclinical IND-enabling studies are underway and planned throughout 2025, with anticipated IND submission in 2H 2026.

*Mayo Clinic Collaboration*

- As announced on December 4, 2024, Solid entered into a collaboration with Mayo Clinic to develop an AAV gene therapy platform for the development of therapies to treat sudden cardiac death-predisposing genetic cardiomyopathies and channelopathies.
- Under the collaboration, Solid received an exclusive license to develop and commercialize six undisclosed cardiac gene therapy programs.

**Platform Technologies – Capsids & Other**
Solid is building an innovative library of enabling technologies, across:

*Capsids & Promoters*

- The Company is building multiple cardiac and neuromuscular capsid and promoter libraries with final capsid selection from the first library anticipated in Q4 2025.
- AAV-SLB101, Solid's proprietary capsid used in SGT-003, has been licensed to 15 academic labs and corporations.

*Immunomodulation*

- Multiple immunomodulatory preclinical studies are underway to determine viability of potential future dosing with AAV-SLB101 in AAV-gene therapy pre-treated and seropositive individuals.

*CMC Purity*

- Solid's manufacturing constructs continue to establish manufacturing excellence with further improvements in full-to-empty capsid ratios seen at research scales in early-stage cardiac programs.

**43rd Annual J.P. Morgan Healthcare Conference Webcast**
Mr. Cumbo and Dr. Brooks will present at the 43rd Annual J.P. Morgan Healthcare Conference on Wednesday, January 15, 2025, at 3:45 p.m. PT (6:45 p.m. ET).

A live webcast of the presentation will be available on the Events page of the Investors section of the Company website or by clicking here. A webcast replay will be archived for 30 days on the Events page.

Institutional investors interested in meeting with management during the conference may reach out to their J.P. Morgan representatives.

**About Solid Biosciences**
Solid Biosciences is a precision genetic medicine company focused on advancing a portfolio of gene therapy candidates including SGT-003 for the treatment of Duchenne muscular dystrophy (Duchenne), SGT-212 for the treatment of Friedreich's ataxia, SGT-501 for the treatment of catecholaminergic polymorphic ventricular tachycardia (CPVT), SGT-601 for the treatment of TNNT2-mediated dilated cardiomyopathy, SGT-401 for

the treatment of BAG3-mediated dilated cardiomyopathy, and additional assets for the treatment of fatal cardiac diseases. Solid is advancing its diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management, and care. Patient-focused and founded by those directly impacted, Solid's mandate is to improve the daily lives of patients living with these devastating diseases. For more information, please visit www.solidbio.com.

**Forward-Looking Statements**

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding future expectations, plans and prospects for the Company; the ability to successfully achieve and execute on the company's goals, priorities and achieve key clinical milestones; the Company's pipeline of programs for neuromuscular and cardiac diseases, including its SGT-003 and SGT-212 programs and expectations for CTA filings, site activations, clinical development, initiation and enrollment in clinical trials, dosing, availability of clinical trial data and potential accelerated approval; the ability to successfully develop other preclinical programs and its capsid libraries; the sufficiency of the Company's cash, cash equivalents, and available-for-sale securities to fund its operations; and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," "working" and similar expressions. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the company's ability to advance SGT-003, SGT-212, SGT-501, SGT-601, SGT-401 and other preclinical programs and capsid libraries on the timelines expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; replicate in clinical trials positive results found in preclinical studies and early-stage clinical trials of the company's product candidates; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne, Friedreich's ataxia and other neuromuscular and cardiac treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-003, SGT-212, SGT-501, SGT-601, SGT-401 and other candidates, achieve its other business objectives and continue as a going concern. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in the company's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the company's views as of the date hereof and should not be relied upon as representing the company's views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

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[1] Unaudited

Source: Solid Biosciences Inc.